5 January 2018
EMA/632132/2017

Public summary of opinion on orphan designation

Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F for the treatment of multiple myeloma

On 16 October 2017, orphan designation (EU/3/17/1925) was granted by the European Commission to GlaxoSmithKline Trading Services Limited, Ireland, for humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F (also known as GSK2857916) for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells are produced in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes uncontrolled, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

What is the estimated number of patients affected by the condition?

At the time of designation, multiple myeloma affected less than 4 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 206,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with corticosteroids to reduce the activity of the body's immune (defence) system. After chemotherapy patients received a stem-cell transplant if they were considered suitable for it. Stem-cell transplantation is a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with multiple myeloma because early studies indicated that patients whose disease came back after several previous treatments responded to treatment with this medicine. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

The abnormal immature plasma cells in patients with multiple myeloma produce a protein on their surface called B-cell maturation antigen (BCMA).

This medicine is made up of MMAF (maleimidocaproyl monomethyl auristatin F), a cytotoxic (cell-killing) molecule, which is attached to a monoclonal antibody (a type of protein) that has been designed to recognise and attach to BCMA. When the medicine is given to the patient, it is expected to attach to BCMA on the myeloma cells and deliver MMAF into the cells. Once inside the myeloma cells, MMAF kills them by interfering with their ability to divide and grow. In addition, by attaching to the myeloma cells the medicine is expected to encourage the immune system (the body's natural defences) to attack them and thereby slow the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with multiple myeloma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for multiple myeloma. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2017 recommending the granting of this designation.

_____

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised monoclonal antibody targeting B-cell maturation antigen conjugated with maleimidocaproyl monomethyl auristatin F	Treatment of multiple myeloma
Bulgarian	Хуманизирано моноклонално антитяло насочено срещу В-клетъчен матурационен антиген, конюгиран с малеймидокапроил монометил ауристатин F	Лечение на мултиплен миелом
Croatian	Humanizirano monoklonsko protutijelo na antigen sazrijevanja B-limfocita konjugirano s maleimidokaproil monometil auristatinom F	Liječenje multiplog mijeloma
Czech	Humanizovaná monoklonální protilátka zacílená na maturační antigen B buněk konjugovaná s maleimidokaproyl monomethyl auristatinem F	Léčba mnohočetného myelomu
Danish	Humant monoklonalt antistof målrettet mod modnet b-celle antigen, som er konjugeret med maleimidocaproylmonomethylauristatin F	Behandling af multipelt myelom
Dutch	Gehumaniseerd monoclonaal antilichaam gericht tegen B-cel maturatie-antigeen, geconjugeerd met maleimidocaproyl monomethylauristatin F	Behandeling van multipel myeloom
Estonian	B-rakkude küpsemise antigeeni vastane humaniseeritud monoklonaalne antikeha, mis on konjugeeritud maleimidokaprüülmonometüülauristatiin F-iga	Multiibelse müeloomi ravi
Finnish	Humanisoitu monoklonaalinen vasta-aine, jonka kohteena on B-solujen kypsymisantigeeni konjugoituna maleiimidokaproyylimonometyyli auristatiini F:ään	Multippeli myelooman hoito
French	Anticorps monoclonal humanisé ciblant l'antigène de maturation des cellules B conjugué au maléimidocaproyl monométhyl auristatine F	Traitement du myélome multiple
German	Humanisierter monoklonaler Antikörper gegen „B Cell Maturation Antigen“ konjugiert mit Maleimidocaproyl-Monomethylauristan F	Behandlung des multiplen Myeloms
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι του αντιγόνου ωρίμανσης Β λεμφοκυττάρων συζευγμένο με μαλεϊμιδοκαπροϋλ μονομεθυλ οριστατίνη F	Θεραπεία πολλαπλού μυελώματος
Hungarian	A B-sejt érési antigént célzó, maleimido-kaproil-monometil-aurisztatin F-fel konjugált monoklonális antitest	Myeloma multiplex kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	Anticorpo umanizzato avente come target l'antigene di maturazione delle cellule B coniugato con maleimidocaproil monometil auristatina F	Trattamento del mieloma multiplo
Latvian	Humanizēta, ar maleimidokaproilmonometilauristatīnu F konjugēta, pret B šūnu nobriešanas antigēnu vērsta monoklonāla antiviela	Multiplās mielomas ārstēšana
Lithuanian	Žmogaus monokloniniai antikūnai, nukriepti prieš B-ląstelių brendimo antigeną, sujungtą su maleimidokaproilmonometilauristatinu F	Dauginės mielomos gydymas
Maltese	Antiġen ta' maturazzjoni ta' ċellola B li jpoġġi fil-mira tiegħu antikorp umanizzat konjugat ma' maleimidocaproyl monomethyl auristatin F	Kura tal-mjeloma multipla
Polish	Humanizowane monoklonalne przeciwciało skierowane przeciwko antygenowi dojrzewania komórek B skoniugowane z maleimidokaproilo-momometylo-aurystatyną F.	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal humanizado dirigido ao antigénio de maturação de células B conjugado com maleimidocaproil monometilauristatina F	Tratamento do mieloma múltiplo
Romanian	Anticorp monoclonal umanizat care vizează antigenul de maturare al celulelor B conjugat cu maleimidocaproil monometil auristatin F	Tratamentul mielomului multiplu
Slovak	Humanizovaná monoklonálna protilátka namierená proti antigénu zrenia B-lymfocytov konjugovaná s maleimidokaproyl monometylauristatín fenylalanínom	Liečba mnohopočetného myelómu
Slovenian	Humanizirano monoklonsko protitelo, usmerjeno proti antigenu dozorevanja B-limfocitov, konjugirano z maleimidokaproil monometil auristatinom F	Zdravljenje multiplega mieloma
Spanish	Anticuerpo monoclonal humanizado frente al antígeno de maduración de Células B conjugado con maleimidocaproil monometil auristatin F	Tratamiento del mieloma múltiple
Swedish	Humaniserad monoklonal antikropp riktad mot B cell maturation antigen konjugerad med maleimidokaproyl monometyl auristatin F	Behandling av multipelt myelom
Norwegian	Humanisert monoklonalt antistoff mot B-celle modningsantigen konjugert med maleimidokaproyl monometyl auristatin F	Behandling av myelomatose
Icelandic	Mannaaðlagað einstofna mótefni gegn B frumu maturation mótefnavaka) tengt maleimídócapró ýl mónómetýl auristatín F	Meðferð við mergfrumuæxli